NON-TECHNICAL ABSTRACT

The overall purpose of this study is to evaluate the possibility of treating Hunter syndrome (mucopolysaccharidosis type II), a severe heritable disease, by a form of gene therapy using an LXSN-class vector "L2SN". This vector is a retrovirus genetically-modified to carry the normal gene for normal human iduronate-2-sulfatase (IDS) which is lacking in patients with Hunter syndrome. For treatment, lymphocyte blood cells will be removed from the patient, grown in the laboratory, and exposed to the L2SN vector. The treated lymphocytes will then be returned to the blood stream by intravenous injection. It is hoped that the treated lymphocytes will survive in the blood stream for several days or longer and will be able to partially replenish the IDS enzyme which is missing. It is hoped that some of the symptoms of Hunter syndrome will be slowed, prevented, or reversed by this treatment.

The specific goals of this study are to:

(1) Determine the amount of IDS enzyme that can be achieved in the body after injecting lymphocytes which have been treated with the L2SN virus.

(2) Determine how long these genetically modified lymphocytes can survive in the blood stream.

(3) Determine if the genetically modified lymphocytes will reduce the abnormal amounts of glycosaminoglycan storage material in urine.

(4) Determine if the genetically modified lymphocytes will decrease the size of the patient's enlarged liver and spleen, and if this treatment will improve heart and respiratory functions.

(5) Determine if there are any other effects of this new form of treatment (i.e., watch for other improvements and for side-effects of the treatment).